The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

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PRESS RELEASE

Committee for Orphan Medicinal Products adopts 100th Positive Opinion on Designation

The twenty-second meeting of the Committee for Orphan Medicinal Products (COMP) was held on 25-26 March 2002. Just two years since the implementation of orphan legislation in the European Union, the Committee celebrated the adoption of its 100th positive opinion recommending orphan medicinal product designation.

Three positive opinions on the designation of orphan medicinal products, were adopted by the Committee during this meeting, for the following conditions:

- Emphysema secondary to congenital alpha-1 antitrypsin deficiency
- Glioma
- Oesophageal cancer

Three oral explanations took place on the first day of the meeting. The COMP noted that 9 applications for orphan medicinal product designation were withdrawn by sponsors.

The European Commission granted 12 decisions on orphan designation[1] since the last COMP meeting on 26 February 2002, see Annex I. The status of orphan designation procedures, as of 26 March 2002, is summarised in the table below:

Intent to file notified	*Applications submitted*	*Applications withdrawn*	*Positive COMP Opinions*	*Negative COMP Opinions*	*Designations granted by Commission*
47	169	43	101	1[2]	98

Further information on designated orphan medicinal products is now publicly available in the form of summarised COMP Opinions[3], which the Agency will routinely publish following adoption of the respective decisions on orphan designation by the European Commission. The first six summarised COMP Opinions were published on the EMEA web-site this month.

The Committee discussed and endorsed a revised version of the Commission guideline (ENTR/6283/00)[4] on the format and content of applications for designation as orphan medicinal products, which has been updated to incorporate comments received during the consultation period. In preparing an application for orphan medicinal product designation, sponsors are requested to follow this guideline.

The 'Points to Consider Document on the Calculation and Reporting of the Prevalence of a Condition for Orphan Designation' (COMP/436/01) was adopted as final by the Committee, following the end of the 3 months consultation period.

The next COMP meeting will be held on 29-30 April 2002.

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1 Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://pharmacos.eudra.org/F2/register/index.htm)

2 Of the five negative opinions adopted to date, one appeal is ongoing. Two applications have been subsequently withdrawn, one has resulted in a final negative decision and one has resulted in a final positive opinion following appeal.

3 Summarised COMP Opinions are available on the EMEA web-site (http://www.emea.eu.int/)

4 ENTR/6283/00 is available on the EMEA and Commission (http://dg3.eudra.org) web-sites

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NOTE: This Press Release, together with other information about the work of the EMEA, may be found on the internet at the following location: http://www.emea.eu.int/

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Medicinal products Designated as Orphan Medicinal Products in March 2002

Active substance	Carmustine (solution for intratumoural injection)
Sponsor	Icon Clinical Research UK Ltd
Orphan Indication	Treatment of glioma
Opinion receipt date	7/1/02
Date of Commission Decision	5/3/02

Active substance	Porfimer sodium (for use with photodynamic therapy)
Sponsor	Axcan Pharma International BV
Orphan Indication	Treatment of high-grade dysplasia in Barrett's Esophagus.
Opinion receipt date	7/1/02
Date of Commission Decision	6/3/02

Active substance	Beclomethasone 17, 21-dipropionate (oral use)
Sponsor	Voisin Consulting
Orphan Indication	Treatment of intestinal graft-versus-host disease
Opinion receipt date	31/1/02
Date of Commission Decision	13/3/02

Active substance	Nitisinone
Sponsor	Swedish Orphan AB
Orphan Indication	Treatment of alkaptonuria
Opinion receipt date	31/1/02
Date of Commission Decision	13/3/02

Active substance	4-(3,5-bis-(hydroxy-phenyl)-1,2,4) triazol-1-yl)-benzoic acid
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of chronic iron overload requiring chelation therapy.
Opinion receipt date	31/1/02
Date of Commission Decision	13/3/02

Active substance	GM-CSF receptor antagonist
Sponsor	British Biotech Pharmaceuticals Ltd
Orphan Indication	Treatment of juvenile myelomonocytic leukaemia
Opinion receipt date	31/1/02
Date of Commission Decision	18/3/02

Active substance	Chimeric IgG monoclonal antibody cG250 for use with ^{131}I
Sponsor	Wilex AG
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	31/1/02
Date of Commission Decision	19/3/02

Active substance	Chimeric IgG monoclonal antibody cG250
Sponsor	Wilex AG
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	31/1/02
Date of Commission Decision	19/3/02

Active substance	Human transferrin conjugated to mutant diphtheria toxin
Sponsor	KS Biomedix Holdings PLC
Orphan Indication	Treatment of glioma
Opinion receipt date	31/1/02
Date of Commission Decision	19/3/02

Active substance	TGF-β2 specific phosphorothioate antisense oligodeoxynucleotide
Sponsor	Antisense Pharma GmbH
Orphan Indication	Treatment of high-grade glioma
Opinion receipt date	31/1/02
Date of Commission Decision	22/3/02

Active substance	Epothilone B
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of ovarian cancer
Opinion receipt date	31/1/02
Date of Commission Decision	22/3/02

Active substance	Humanized anti-KSA monoclonal antibody-human interleukin-2 fusion protein
Sponsor	Merck KgaA
Orphan Indication	Treatment of renal cell carcinoma
Opinion receipt date	31/1/02
Date of Commission Decision	22/3/02